

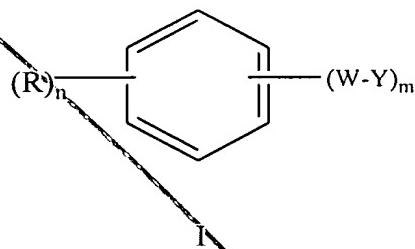
Sus A1

What is claimed is:

1. A method for treating a disease or condition modulated by protein expression in a mammal suffering from, susceptible to, or recovering from the disease or condition, the method comprising administering to the mammal a therapeutically effective amount of at least one carbocyclic aryl compound having spaced from the aryl ring a substituent of carboxy acid, ester, sulfonic acid, nitro, cyano or haloalkyl; or a pharmaceutically acceptable salt thereof.
2. The method of claim 1 wherein the substituent is spaced from the carbocyclic aryl ring by an unsaturated carbon chain.
3. The method of claim 1 wherein the substituent is spaced from the carbocyclic aryl ring by a C₂₋₆alkenylene chain.
4. The method of claim 1 wherein the substituent is carboxy.
5. The method of claim 1 wherein the compound is of the following

Formula I:

Sus A2



wherein each W is independently optionally substituted alkylene; optionally substituted alkenylene; optionally substituted alkynylene; optionally substituted heteroalkylene; optionally substituted heteroalkenylene; or optionally substituted heteroalkynynylene;

*Su b A2
cont.*

each Y is independently a carboxy acid, ester, sulfonic acid, nitro, cyano or haloalkyl;

each R is independently halogen, cyano, nitro, optionally substituted alkyl; optionally substituted alkenyl; optionally substituted alkynyl; optionally substituted alkoxy; optionally substituted alkylthio; optionally substituted alkylsulfinyl; optionally substituted alkylsulfonyl; optionally substituted carbocyclic aryl; optionally substituted aralkyl;

m is an integer of from 1 to 6; n is an integer of from 0 to 5; and pharmaceutically acceptable salts thereof, with the exclusion of 4-phenylbutyric acid.

*Sub B1
Cont*

6. The method of claim 5, wherein the compound comprises a straight carbon chain of about four carbon atoms.

*Sub B2
Cont*

7. The method of claim 6, wherein the compound comprises a carbon-carbon double bond in the second ($\Delta 2$) or third ($\Delta 3$) position of the chain.

*Sub B3
Cont*

8. The method of claim 7, wherein the compound further comprises a phenyl ring in the fourth position of the chain.

*Sub B1
Cont*

9. The method of claim 8, wherein the compound is a cis or trans stereoisomer.

*Sub B1
Cont*

10. The method of claim 9, wherein the compound is 4-phenyl- $\Delta 3$ -transbutenoic acid; or a pharmaceutically acceptable salt thereof.

*Sub B1
Cont*

11. The method of claim 1 wherein the disease or condition impacted by the protein expression afflicts or is suspected of afflicting the nervous, hepatic, or respiratory system.

*Sub B1
Cont*

12. The method of claim 11, wherein the respiratory system disease or condition is associated with abnormal lung function.

- Sub A4*
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Cont*
13. The method of claim 12, wherein the abnormal lung function is associated with a incorrect surfactant protein expression.
 14. The method of claim 13, wherein the surfactant protein is surfactant protein C.
 15. The method of claim 12, wherein the abnormal lung function is associated with incorrect protein expression of a transmembrane protein.
 16. The method of claim 15, wherein the respiratory disease is cystic fibrosis (CF) and the transmembrane protein is the cystic fibrosis transmembrane regulator (CFTR).
 17. The method of claim 11, wherein the hepatic disease or condition is associated with the liver.
 18. The method of claim 17, wherein the disease or condition is α 1 anti-trypsin disease.
 19. The method of claim 11, wherein the nervous system disease or condition is associated with the brain.
 20. The method of claim 19, wherein the abnormal brain disease or condition is Alzheimer's disease or infection by a virus or prion.
 21. The method of claim 1, wherein the disease or condition is Marfan syndrome, familial hypercholesterolemia, or Tay-Sachs disease.
 22. The method of claim 1, wherein the compound is administered to the mammal by a stent, needle or in a solid dosage form.
 23. The method of claim 23, wherein the compound is administered to the mammal orally, intramuscularly or intraperitoneally.

Sub A4 cont.

24. A method for treating a mammal suffering from, susceptible to, or recovering from cystic fibrosis (CF), the method comprising administering to the mammal a therapeutically effective amount of at least one carbocyclic aryl compound having spaced from the aryl ring a substituent of carboxy acid, ester, sulfonic acid, nitro, cyano or haloalkyl compound; or a pharmaceutically acceptable salt thereof.

25. The method of any one of claims 1-24, wherein the compound increases or decreases expression of a subject protein by at least about 10% in a standard *in vitro* assay for measuring the subject protein.

Sub A5 Cont

26. The method of claim 25, wherein the subject protein is one or more of heat shock protein 70 (hsc70) or the cystic fibrosis transmembrane regulator (CFTR).

27. The method of claim 26, wherein the compound is 4-phenyl- Δ 3-transbutenoic acid, 4-phenyl- Δ 2-transbutenoic acid; or a pharmaceutically acceptable salt thereof.

28. The method of claim 27, wherein the pharmaceutically acceptable salt comprises 4-phenyl- Δ 3-transbutenoate or 4-phenyl- Δ 2-transbutenoate.

28. The method of any one of claims 1-24, wherein the compound has an IC₅₀ of at least about 0.001 to about 100 μ M in a standard *in vitro* assay for measuring the subject protein.

Sub A5

29. The method of claim 29, wherein the compound exhibits an IC₅₀ of about 100 μ m or less in the assay.

29. The method of claim 28, wherein the subject protein is one or more of heat shock protein 70 (hsc70).

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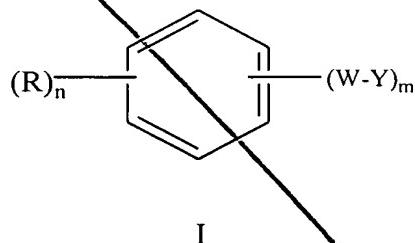
30. The method of claim 29, wherein the compound is 4-phenyl- Δ 3-transbutenoic acid, 4-phenyl- Δ 2-transbutenoic acid; or a pharmaceutically acceptable salt thereof.

31. The method of claim 30, wherein the pharmaceutically acceptable salt comprises 4-phenyl- Δ 3-transbutenoate or 4-phenyl- Δ 2-transbutenoate.

32. The method of claim 1, wherein the mammal is a primate.

33. The method of claim 32, wherein the primate is a human subject.

34. A method for treating a human subject suffering from, susceptible to, or recovering from a disease or condition associated with surfactant protein C, cystic fibrosis (CF), α 1 anti-trypsin disease, Alzheimer's disease, Marfan syndrome, familial hypercholesterolemia, or Tay-Sachs disease, the method comprising administering to the human subject a therapeutically effective amount of compound is of the following Formula I:



wherein each W is independently optionally substituted alkylene; optionally substituted alkenylene; optionally substituted alkynylene; optionally substituted heteroalkylene; optionally substituted heteroalkenylene; or optionally substituted heteroalkynynylene;

each Y is independently a carboxy acid, ester, sulfonic acid, nitro, cyano or haloalkyl;

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Cont.

each R is independently halogen, cyano, nitro, optionally substituted alkyl; optionally substituted alkenyl; optionally substituted alkynyl; optionally substituted alkoxy; optionally substituted alkylthio; optionally substituted alkylsulfinyl; optionally substituted alkylsulfonyl; optionally substituted carbocyclic aryl; optionally substituted aralkyl;

m is an integer of from 1 to 6; n is an integer of from 0 to 5; and pharmaceutically acceptable salts thereof, with the exclusion of 4-phenylbutyric acid.

35. A method for determining the therapeutic capacity of a compound for treating a disease or disorder modulated by protein expression, the method comprising,

- 1) culturing a population of cells capable of expressing hsc70,
- 2) adding at least one known or candidate compound to the cells;
- 3) measuring at least one step capable of increasing or decreasing the protein expression; and
- 4) determining the effect of the known or candidate compound on the expression of at least one subject protein.

36. The method of claim 35, wherein the step measured by the method is at least transcription of the subject protein.

37. The method of claim 35, wherein the step measured by the method is at least trafficking of the subject protein.

38. The method of claim 37, wherein the measured step further comprises measuring levels of the subject protein immunologically.

39. The method of claim 38, wherein the method further comprises an ELISA detection of the subject protein.

40. The method of claim 1, wherein the compound is further administered to prevent the disease or condition.

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contd*

41. A kit for performing the method of claim 1, wherein the kit comprises a container means comprising at least one of the compounds.

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